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Phenylketonuria (PKU) (mRNA-3283)

Last updated: December 6, 2018

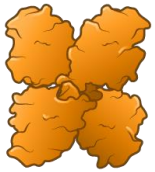
Modality	Program #	Program *Indication*	Preclinical development	Phase 1	Phase 2	Phase 3 and commercial	Moderna rights
Systemic intracellular therapeutics	mRNA-3704	MUT *Methylmalonic acidemia, MMA*					Worldwide
	mRNA-3927	PCCA+PCCB *Propionic acidemia, PA*					Worldwide
	mRNA-3283	PAH *Phenylketonuria, PKU*					Worldwide

mRNA-3283 is in IND-enabling GLP toxicology studies

Phenylketonuria (PKU) overview

Opportunity to address unmet need

- PKU is a rare inherited metabolic disease resulting from a deficiency in the metabolism of phenylalanine, or PHE, due to mutations within the enzyme phenylalanine hydroxylase, PAH
- **Disease burden:** Progressive mental disability from tyrosine deprivation
- **Target population:** Incidence of ~1:10K-15K live births in the US
  - ~21,000-32,000 patients in the US
- Approved therapies include:
  - 20-56% of patients respond favorably to BioMarin's Kuvan
  - Nonresponsive patients treated with restricted diet
  - Biomarin's Palynziq was approved in May 2018



Phenylalanine hydroxylase (PAH) homotetramer

Moderna concept: IV-administered mRNA encoding PAH enzyme to restore deficient or defective intracellular enzyme activity

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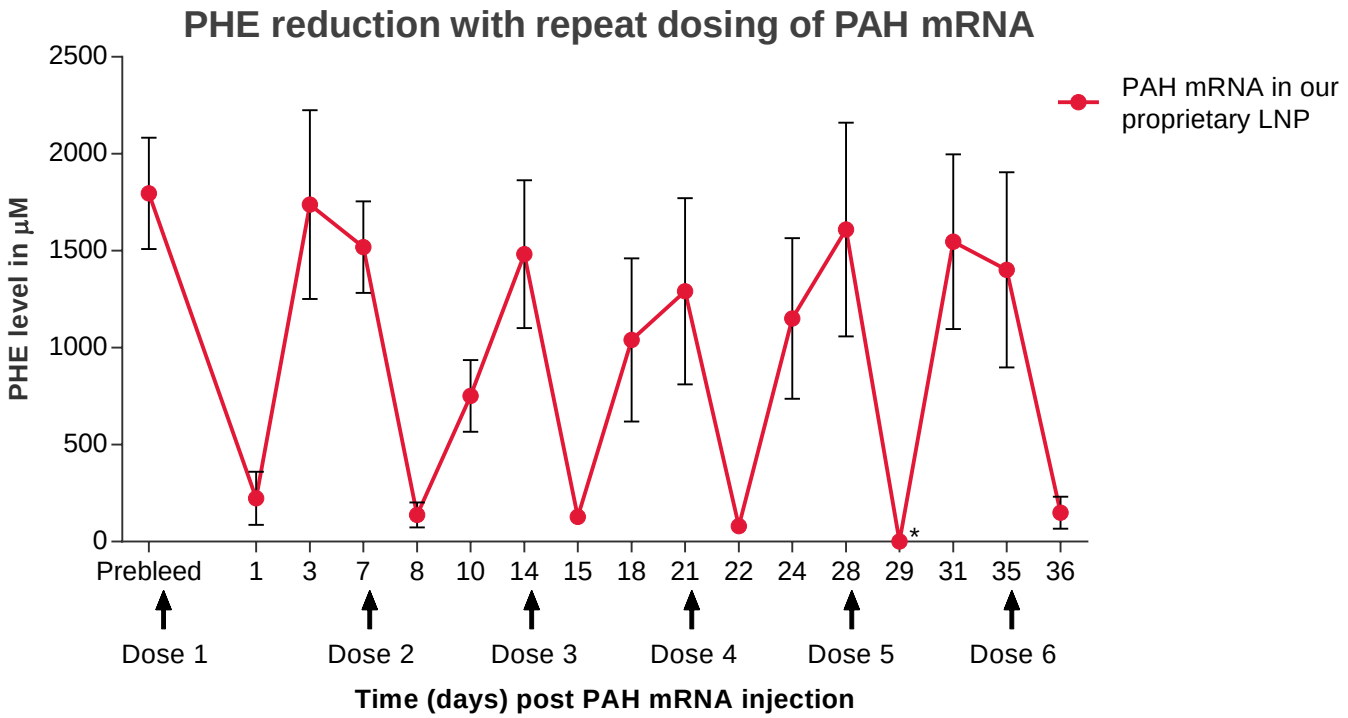
Pre-clinical data – restoration of enzymatic activity

Study Design:

Species: **Mouse**

- Animals: PAH-/-
- Dose: 0.5 mpk
- Dosing Schedule: weekly
- Injection Route: IV
- Sample Size: [5-6]



We have demonstrated the ability to impact PHE levels with repeat dosing of our mRNA in pre-clinical studies

*Data not collected due to a snow storm

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This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended including, but not limited to, statements concerning potential development candidate applications, development candidate activities, preclinical and clinical studies, regulatory submissions and approvals, risk management and estimates and forward-looking projections with respect to Moderna or its anticipated future performance or events. In some cases, forward-looking statements can be identified by terminology such as “may,” “should,” “expects,” “intends,” “plans,” “aims,” “anticipates,” “believes,” “estimates,” “predicts,” “potential,” “continue,” or the negative of these terms or other comparable terminology, although not all forward-looking statements contain these words. The forward-looking statements in this presentation are neither promises nor guarantees, and you should not place undue reliance on these forward-looking statements because they involve known and unknown risks, uncertainties and other factors, many of which are beyond Moderna’s control and which could cause actual results to differ materially from those expressed or implied by these forward-looking statements. These risks, uncertainties and other factors include, among others: preclinical and clinical development is lengthy and uncertain, especially for a new category of medicines such as mRNA, and therefore Moderna’s preclinical programs or development candidates may be delayed, terminated, or may never advance to or in the clinic; no mRNA drug has been approved in this new potential category of medicines, and may never be approved; mRNA drug development has substantial clinical development and regulatory risks due to the novel and unprecedented nature of this new category of medicines; and those described in Moderna’s Prospectus filed with the U.S. Securities and Exchange Commission (SEC) on December 7, 2018 and in subsequent filings made by Moderna with SEC, which are available on the SEC's website at www.sec.gov. Except as required by law, Moderna disclaims any intention or responsibility for updating or revising any forward-looking statements in this presentation in the event of new information, future developments or otherwise. These forward-looking statements are based on Moderna’s current expectations and speak only as of the date hereof.

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